Observational study evaluating the long-term safety and efficacy of avapritinib in the first-line treatment of patients with platelet-derived growth factor receptor alpha (PDGFRA) D842V-mutated gastrointestinal stromal tumour (GIST)

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Study Ongoing

Administrative details

EU PAS number

EUPAS41969

Study ID

43762

DARWIN EU® study

No

Study countries

- [ ] Italy
- [ ] Netherlands
- [ ] Spain
- [ ] United States

Study description

This study protocol concerns a non-interventional observational study evaluating the long-term safety (primary objective) and efficacy (secondary objective) of avapritinib in the first-line treatment of patients with platelet-derived growth factor receptor alpha (PDGFRA) D842V mutated gastrointestinal stromal tumour (GIST), (or following ≤4 months of imatinib treatment). This study is an imposed PASS (category 2) as part of the CMA of avapritinib.

Study status

Ongoing

Research institutions and networks

Institutions

Blueprint Medicines

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Institution

Contact details

Study institution contact

Medical Information

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Study contact

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Primary lead investigator

Alison Doane

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/08/2021

Actual: 31/08/2021

Study start date

Planned: 31/12/2022

Actual: 31/03/2023

Data analysis start date

Planned: 15/10/2026

Date of interim report, if expected

Planned: 28/02/2025

Date of final study report

Planned: 31/03/2027

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Blueprint Medicines Netherlands B.V.

Study protocol

blu-285-1406 pass protocol v2.0.pdf(1.4 MB)

BLU-285-1406 PASS Protocol v5.0.pdf(1.14 MB)

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 2 (specific obligation of marketing authorisation)

Regulatory procedure number

EMEA/H/C/PSP/S/0092.1

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

The overall objective is to collect long-term safety and efficacy data for avapritinib in first-line patients with PDGFRA D842V-mutated GIST, (or following ≤4 months of imatinib treatment).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

AYVAKYT

Study drug International non-proprietary name (INN) or common name

AVAPRITINIB

Anatomical Therapeutic Chemical (ATC) code

(L01EX18) avapritinib

avapritinib

Medical condition to be studied

Gastrointestinal stromal tumour

Additional medical condition(s)

Platelet-derived growth factor receptor alpha (PDGFRA) D842V-mutated gastrointestinal stromal tumour (GIST)

Population studied

Age groups

Adult and elderly population (≥18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (≥ 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

50

Study design details

Outcomes

To describe types, severity and rates of AEs, SAEs, AEs leading to discontinuation or decreased dosing of avapritinib, AESIs, and deaths.
This PASS has been implemented following a commitment to the EMA to address the Specific Obligation of the CMA in Europe to provide additional safety data for patients with PDGFRA D842V-mutated GIST on first-line avapritinib treatment, (or≤4 months of imatinib),
To evaluate efficacy in terms of disease response to treatment, PFS and OS as well as duration of treatment and duration of response.

Data analysis plan

Analyses will be performed on the safety population, i.e. enrolled patients who received at least one dose of avapritinib.
For the primary endpoint, the number, proportions and incidence rate of patients experiencing an AE (overall and by SOC/PT terms), and the number, proportions of patients with avapritinib treatment changes due to an AE (overall and by comorbidity) will be determined.
For the secondary endpoints, the following will be determined: 1) overall survival (OS), progression-free survival, treatment duration, tumor response and response duration by the Kaplan-Meier method, 2) OS rates at 12- and 24-months, and 3) overall response rates.
Inviting consecutive patients at sites and retrospective patients who discontinued treatment will minimize selection bias.
A statistical analysis plan will be developed before the first data lock point, which will detail handling of missing data, correction of inconsistencies or errors, and differences in outcome definitions.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection. Data will be analysed from treatment-naïve patients who will be newly enrolled into this study, and from patients who meet the eligibility criteria and already received first-line avapritinib during participation in study BLU-285-1101 or CUP/EAP.

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No